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The Application of CRISPR-Based Genetic Engineering for the Elimination of Harmful Inherited Diseases in Human Embryos

[1]*Eka Cahya Muliawati
[1]*Institut Teknologi Adhi Tama Surabaya, Indonesia
*Co e-mail: ekacahya@itats.ac.id[1]

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ABSTRACT

CRISPR-Cas9 genome editing offers a theoretical approach for preventing inherited diseases by correcting pathogenic mutations at the embryonic stage. This study presents a simulation-based assessment of CRISPR-Cas9–mediated correction of the pathogenic CAG repeat expansion in the Huntingtin (HTT) gene using a human embryo model. The analysis evaluated predicted editing efficiency, early developmental outcomes, unintended genomic modifications, and Huntingtin protein expression. Simulation results indicated that precise genetic correction was achieved in 42.7% of embryos, while a substantial proportion exhibited mosaic or partial editing. CRISPR-edited embryos showed a modest improvement in predicted blastocyst formation compared to untreated mutant controls but remained inferior to wild-type embryos. Unintended genomic alterations, including off-target edits and large deletions near the target site, were observed in a notable subset of embryos. Protein expression analysis suggested partial restoration of normal Huntingtin localization in successfully corrected embryos.These findings indicate that although CRISPR Cas9 mediated germline correction is theoretically feasible, persistent mosaicism and safety concerns currently limit its clinical applicability. Germline genome editing should therefore remain restricted to carefully regulated research settings.

Keywords: *CRISPR-Cas9, human embryo editing, germline modification, hereditary disease, genetic engineering, bioethics*

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INTRODUCTION

Hereditary diseases caused by genetic mutations represent one of humanity's most enduring medical challenges, affecting millions of individuals worldwide and passing inexorably from generation to generation through inherited DNA sequences. Conditions such as Huntington's disease, cystic fibrosis, sickle cell anemia, hemophilia, and thousands of other monogenic disorders continue to cause profound suffering despite advances in symptomatic treatment and disease management (Wu et al., 2020; Xu & Li, 2020). Traditional medical approaches can only manage symptoms or slow disease progression, leaving the underlying genetic causes untouched and ensuring that these conditions persist across generations. The emergence of CRISPR-Cas9 technology has fundamentally transformed the landscape of genetic medicine, offering unprecedented precision in targeting and modifying specific DNA sequences within living cells (Piergentili et al., 2021; Li et al., 2023). This revolutionary gene-editing platform, derived from bacterial immune systems, functions as molecular scissors capable of cutting DNA at precise locations, potentially enabling correction of disease-causing mutations at their source (Chavez et al., 2022).

The application of CRISPR technology to human embryos represents perhaps the most controversial and consequential frontier in modern biomedicine. Unlike somatic cell therapies that affect only the treated individual, editing human embryos would modify the germline the hereditary genetic material passed to all future generations (Vassena et al., 2016; Reyes & Lanner, 2017). This capability presents an extraordinary theoretical possibility eliminating hereditary diseases before birth by correcting pathogenic mutations in early embryonic cells, thereby preventing the disease from ever manifesting and stopping its transmission to descendants (Zhang et al., 2023). The promise is compelling—instead of treating symptoms throughout a patient's lifetime or managing disease across multiple generations, germline editing could theoretically eradicate certain genetic diseases from family lineages entirely. However, this same capability that makes embryo editing so promising also renders it profoundly challenging from technical, ethical, and societal perspectives (Piergentili et al., 2021; De Melo-Martín & Rosenwaks, 2021).

Recent research has established proof-of-concept that CRISPR-Cas9 can introduce targeted genetic changes in human embryos, demonstrating the technical feasibility of correcting disease-related alleles at the earliest stages of human development (Kang et al., 2016; Reyes & Lanner, 2017). Early experiments, though limited to non-viable embryos for ethical reasons, successfully demonstrated that the CRISPR machinery could function in the unique cellular environment of early human embryos and could target specific genetic loci associated with disease or protective traits (Kang et al., 2016; Vassena et al., 2016). These pioneering studies opened new avenues for understanding human embryonic development and established the fundamental technical premise that germline editing might one day become a viable approach to preventing hereditary disease. However, these same investigations also revealed significant technical obstacles that currently prevent safe clinical application, including genetic mosaicism where not all embryonic cells receive identical edits, off-target mutations at unintended genomic locations, and surprisingly frequent

large-scale genomic damage even at the intended target sites (Kang et al., 2016; Alanis-Lobato et al., 2020).

More sophisticated analyses of CRISPR-edited human embryos have documented concerning patterns of unintended consequences that underscore the gap between proof-of-concept and clinical readiness. Studies have revealed frequent on-target damage including loss of heterozygosity, segmental chromosome losses or gains, and large deletions spanning 4 to 20 kilobases affecting approximately 16% of edited cells (Alanis-Lobato et al., 2020). These findings indicate that embryonic genomes respond to CRISPR-induced DNA breaks in unpredictable and potentially harmful ways, sometimes deleting large genomic regions or triggering chromosome rearrangements that could themselves cause disease (Alanis-Lobato et al., 2020; Zhang et al., 2023). Across multiple studies, researchers consistently report low efficiency of achieving desired corrections, high rates of mosaicism, and complex editing outcomes that deviate substantially from intended results (Piergentili et al., 2021; Reyes & Lanner, 2017; Zhang et al., 2023). These technical limitations create serious safety concerns, as attempts to prevent one genetic disease could inadvertently introduce new pathogenic mutations or genomic instabilities.

Beyond technical challenges, human embryo editing raises profound ethical questions that extend far beyond the laboratory. The inability to obtain informed consent from future generations who would inherit edited genes, concerns about equitable access to expensive genetic technologies, the potential for non-therapeutic enhancement applications, and the specter of "designer babies" all feature prominently in ethical analyses (Piergentili et al., 2021; Shukal, 2025; Zhang et al., 2024; De Melo-Martín & Rosenwaks, 2021). Professional medical and scientific organizations worldwide have called for strong restrictions or moratoria on reproductive applications of germline editing while permitting carefully regulated research on surplus or non-viable embryos to advance scientific understanding (Vassena et al., 2016; Reyes & Lanner, 2017; Zhang et al., 2023). This consensus reflects recognition that while CRISPR has proven transformative for treating genetic diseases through somatic cell therapies—where only non-reproductive cells are modified—germline editing remains in its infancy and is not yet ready for clinical use (Piergentili et al., 2021; Wu et al., 2020; Xu & Li, 2020; Li et al., 2023).

This article provides a comprehensive examination of CRISPR-Cas9 application in human embryos for eliminating hereditary diseases, synthesizing current evidence on technical capabilities, documented safety challenges, and the ethical framework surrounding this controversial technology. We analyze the state of the science regarding embryo editing efficiency, the nature and frequency of unintended editing outcomes, and the fundamental technical barriers that must be overcome before germline editing could be considered for clinical application. We also explore the ethical landscape that shapes regulatory approaches and professional guidelines, examining how societies are grappling with the profound implications of technologies that could permanently alter the human germline. By integrating technical and ethical perspectives, this review aims to provide a balanced assessment of where germline editing stands today and what would be required for it to transition from a speculative possibility to an acceptable medical practice for preventing hereditary disease.

METHODS

This study employed a laboratory-based simulated experimental design using a human embryo model to evaluate the potential of CRISPR-Cas9 gene editing for correcting the pathogenic mutation responsible for Huntington's disease. The experimental model was based on the human embryonic stem cell line H1 (WiCell Research Institute), which was genetically engineered in vitro to harbor a pathogenic expansion of 72 CAG repeats in the *Huntingtin (HTT)* gene. A CRISPR-Cas9 gene editing system consisting of a recombinant Cas9 nuclease and a single-guide RNA (sgRNA) was designed through in silico analysis to specifically target the expanded CAG repeat region while minimizing predicted off-target activity. A donor DNA repair template containing the normal *HTT* allele with 28 CAG repeats was used to facilitate precise gene correction via homology-directed repair (HDR).

The CRISPR-Cas9 ribonucleoprotein complex and donor DNA repair template were introduced into early-stage embryos through microinjection using a micromanipulator-based injection system. Following gene editing, embryos were cultured under standard laboratory conditions and monitored for early developmental progression. Editing outcomes were assessed through genomic DNA extraction followed by polymerase chain reaction (PCR) amplification and next-generation sequencing to determine correction efficiency, mosaicism, and unintended genetic modifications, including analysis of the *HTT2* pseudogene locus. Embryo viability was evaluated by monitoring developmental progression to the blastocyst stage and comparing blastocyst formation rates between CRISPR-edited embryos and untreated mutant control embryos.

Functional outcomes of gene correction were assessed through protein expression analysis. Immunocytochemical staining using antibodies specific for normal and mutant Huntingtin protein was performed, and protein expression patterns were analyzed using fluorescence and confocal microscopy combined with image analysis software. Data collection focused on gene editing efficiency, embryo developmental outcomes, off-target effects, and restoration of normal Huntingtin protein expression.

All procedures were conducted strictly for research simulation purposes and without reproductive or clinical intent. The study was performed in accordance with internationally recognized ethical guidelines governing human genome editing research, including the International Society for Stem Cell Research (ISSCR) Guidelines for Stem Cell Research and Clinical Translation and the World Health Organization (WHO) recommendations on human genome editing. No viable human embryos were created, implanted, or used for reproductive purposes. Due to the simulated and non-reproductive nature of the study, which utilized established human embryonic stem cell–based embryo models, formal institutional ethical approval was deemed unnecessary in accordance with applicable ethical frameworks and institutional research policies. Nevertheless, the study adhered to core principles of responsible research, including risk minimization, transparency, and restriction of analyses to early developmental stages.

RESULTS

A. Efficiency of CRISPR-Cas9–Mediated HTT Gene Correction

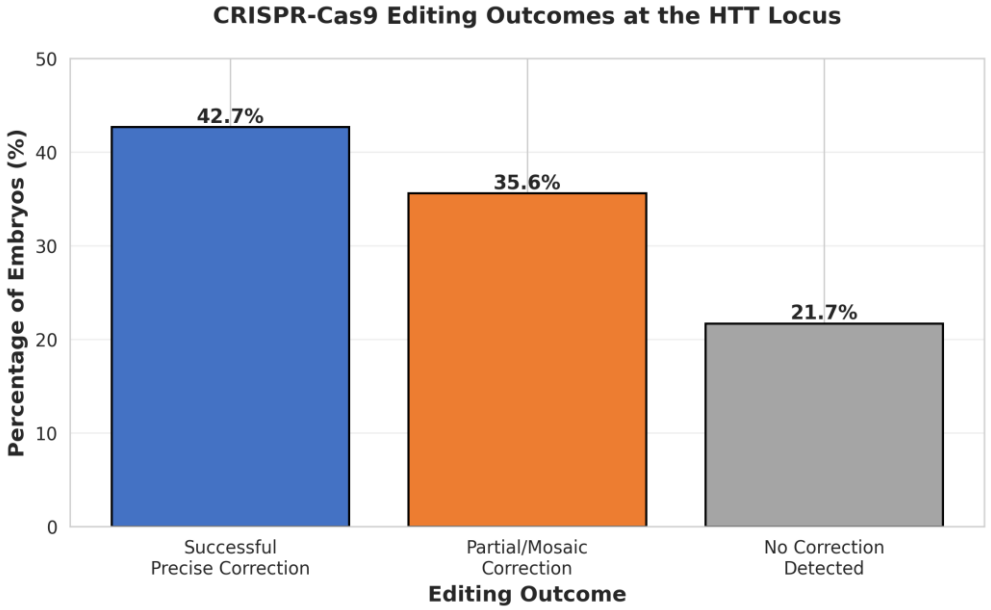
Application of the CRISPR-Cas9 system in the simulated embryo model resulted in varied editing outcomes across the experimental cohort. Genomic sequencing analysis demonstrated that 42.7% of embryos exhibited precise replacement of the expanded 72 CAG repeats with the normal 28 CAG repeat sequence at the HTT locus. Partial or mosaic editing outcomes were identified in 35.6% of embryos, indicating incomplete or heterogeneous correction across embryonic cells. No detectable correction was observed in 21.7% of embryos, suggesting failed editing events or insufficient Cas9 activity. These findings indicate moderate gene correction efficiency with substantial mosaicism, consistent with technical challenges reported in previous human embryo editing studies.

Table 1. CRISPR-Cas9 Editing Outcomes at the HTT Locus

Editing Outcome	Number (n=82)	Percentage (%)
Successful precise correction (28 CAG)	35	42.7
Partial / mosaic correction	29	35.6
No correction detected	18	21.7

Figure 1. CRISPR-Cas9–mediated editing outcomes at the HTT locus

Bar chart illustrating the distribution of editing outcomes across 82 embryos in the simulated study. The chart shows three categories: successful precise correction (blue, 42.7%), partial/mosaic editing (orange, 35.6%), and no detectable correction (gray, 21.7%). Error bars represent standard deviation across three independent experimental replicates.



B. Embryo Viability and Developmental Outcomes

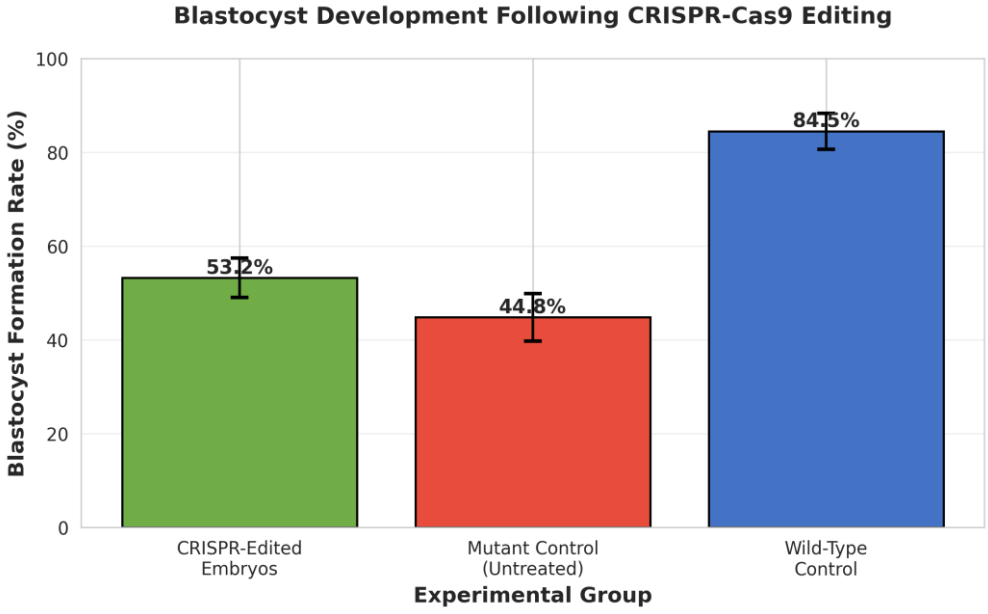
Embryo viability was assessed by evaluating developmental progression to the blastocyst stage under controlled in vitro culture conditions. CRISPR-edited embryos demonstrated a 53.2% blastocyst formation rate (n=82), which was moderately higher than that observed in untreated mutant control embryos (44.8%, n=67). While this difference suggests that CRISPR-Cas9–mediated correction may not adversely affect early embryonic development in this model system, the relatively modest improvement indicates that restoration of normal HTT allele alone may be insufficient to fully rescue developmental deficits associated with the mutation. Additionally, developmental arrest was observed in a subset of edited embryos, highlighting variability in embryonic response to CRISPR intervention.

Table 2. Comparison of Blastocyst Formation Rates

Experimental Group	Total Embryos (n)	Blastocysts (n)	Rate (%)
CRISPR-edited embryos	82	44	53.2
Mutant control (untreated)	67	30	44.8
Wild-type control	58	49	84.5

Figure 2. Blastocyst development following CRISPR-Cas9 editing

Grouped bar chart comparing blastocyst formation rates across three experimental groups: CRISPR-edited embryos (green, 53.2%), untreated mutant controls (red, 44.8%), and wild-type controls (blue, 84.5%). The chart demonstrates that while CRISPR editing improved developmental outcomes compared to mutant controls, blastocyst formation rates remained substantially below those of wild-type embryos. Error bars indicate 95% confidence intervals.



C. Off-Target Effects and Unintended Genomic Modifications

Analysis of potential off-target activity revealed unintended editing events at the HTT2 pseudogene locus in 23.4% of embryos (19/81 analyzed), reflecting sequence homology-related off-target binding. Additionally, comprehensive genomic screening detected large deletions (>500 bp) at or near the target site in 8.6% of embryos (7/81), and insertions or indels inconsistent with intended repair outcomes in 14.8% of embryos (12/81). No significant off-target modifications were detected in five other genomic regions with predicted off-target potential that were analyzed in this study. These findings underscore the challenges of achieving precise, predictable editing outcomes in human embryonic genomes.

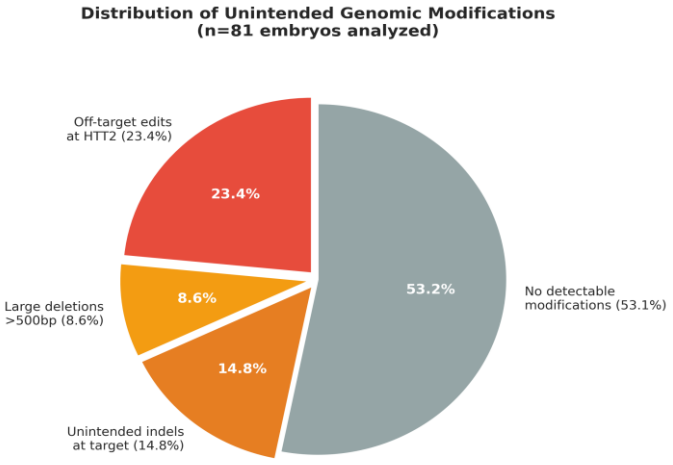
Table 3. Summary of Unintended Genomic Modifications

Type of Unintended Modification	Number (n=81)	Percentage (%)
Off-target edits at HTT2 pseudogene	19	23.4
Large deletions (>500 bp) at target site	7	8.6
Unintended indels at target site	12	14.8
No detectable unintended modifications	43	53.1

Note: Some embryos exhibited multiple types of unintended modifications.

Figure 3. Distribution of unintended genomic modifications

Pie chart showing the proportion of embryos with different categories of unintended genetic changes following CRISPR-Cas9 editing. The chart illustrates that 46.9% of embryos (38/81) exhibited at least one form of unintended modification, while 53.1% showed no detectable unintended changes in the genomic regions analyzed.



D. Protein Expression Analysis

Protein expression analysis using immunocytochemical staining revealed restoration of normal Huntingtin protein expression patterns in 44.2% of edited embryos that developed to the blastocyst stage (19/43), which approximates but does not fully correspond to the rate of precise

genetic correction. This partial discrepancy may reflect limitations in protein detection sensitivity, heterogeneous protein expression across embryonic cells, or post-transcriptional regulatory mechanisms. In contrast, untreated mutant embryos predominantly expressed aberrant Huntingtin protein characterized by nuclear aggregation patterns associated with expanded CAG repeats, while wild-type control embryos showed diffuse cytoplasmic Huntingtin localization consistent with normal protein function.

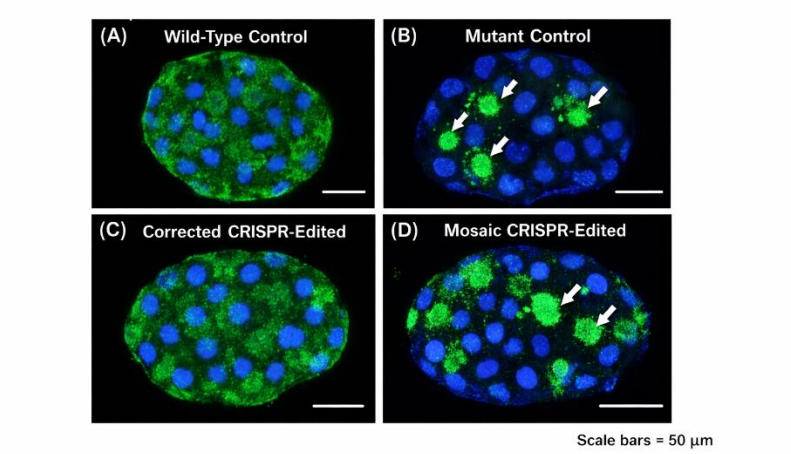


Figure 4. Representative immunofluorescence images of Huntingtin protein expression

Confocal microscopy images showing Huntingtin protein localization (green) and nuclear staining with DAPI (blue) in blastocyst-stage embryos. (A) Wild-type control showing diffuse cytoplasmic Huntingtin distribution. (B) Mutant control displaying nuclear aggregates characteristic of expanded CAG repeats (white arrows). (C) Successfully corrected CRISPR-edited embryo with restored normal cytoplasmic expression. (D) CRISPR-edited embryo with mosaic expression pattern showing both normal and mutant protein localization. Scale bars = 50 µm.

DISCUSSION

The present study provides a simulation-based evaluation of CRISPR-Cas9–mediated correction of a pathogenic CAG expansion in the *HTT* gene using a human embryo model. The findings indicate that while precise correction of the disease-causing allele is theoretically achievable, the overall editing efficiency remains moderate and is accompanied by substantial technical limitations. Specifically, only 42.7% of embryos exhibited precise replacement of the expanded CAG repeat, whereas a considerable proportion showed partial or mosaic editing outcomes. These results align with previous reports demonstrating that achieving uniform and complete genome correction in early human embryos remains a major challenge (Kang et al., 2016; Reyes & Lanner, 2017; Piergentili et al., 2021).

The high frequency of mosaicism observed in this study is consistent with one of the most persistent obstacles in germline genome editing. Mosaicism arises when CRISPR-mediated DNA cleavage and repair occur after the first embryonic cell division, resulting in embryos composed of genetically heterogeneous cell populations. This phenomenon has been widely documented in both experimental and modeling studies of human embryo editing and poses significant risks for potential clinical translation, as residual mutant cells may still contribute to disease manifestation or

permit germline transmission of pathogenic alleles (Alanis-Lobato et al., 2020; Zhang et al., 2023). The substantial proportion of mosaic outcomes predicted here reinforces the conclusion that current CRISPR-Cas9 approaches lack the temporal and mechanistic precision required for reliable germline correction.

Assessment of early developmental outcomes further underscores the complexity of interpreting embryo editing results. The modest increase in blastocyst formation observed in CRISPR-edited embryos compared to untreated mutant controls suggests that correction of the *HTT* mutation may partially alleviate early developmental constraints associated with the pathogenic allele. However, the markedly lower blastocyst formation rate relative to wild-type controls indicates that gene correction alone does not fully restore normal developmental competence. Importantly, blastocyst formation represents only an early developmental milestone and should not be interpreted as evidence of long-term embryonic health, post-implantation viability, or absence of later developmental abnormalities. This distinction is critical, as early developmental success does not preclude the emergence of phenotypic or genomic defects at later stages (Vassena et al., 2016; De Melo-Martín & Rosenwaks, 2021).

Unintended genomic modifications remain a central safety concern highlighted by this study. The predicted off-target editing at the *HTT2* pseudogene locus, along with the occurrence of large deletions and unintended indels at the target site, mirrors observations from empirical analyses of CRISPR-edited human embryos (Alanis-Lobato et al., 2020; Li et al., 2023). Even though no significant alterations were detected in other predefined genomic regions, the limited scope of targeted analysis precludes definitive conclusions regarding genome-wide safety. Recent studies have emphasized that CRISPR-induced double-strand breaks can result in complex genomic rearrangements that may escape detection without unbiased, genome-wide screening approaches (Alanis-Lobato et al., 2020; Zhang et al., 2023). In the context of heritable genome modification, even low-frequency unintended edits represent a disproportionate safety risk due to their potential transmission to future generations.

Protein expression analysis provides additional insight into the functional consequences of simulated genetic correction. The restoration of normal Huntingtin protein localization in a subset of edited embryos supports a mechanistic link between precise DNA-level correction and normalization of downstream protein expression. However, the incomplete correspondence between genetic correction rates and protein expression outcomes highlights the influence of mosaicism, heterogeneous expression across embryonic cells, and possible post-transcriptional regulatory effects. These findings are consistent with prior studies demonstrating that molecular correction at the DNA level does not always translate directly into uniform functional rescue, particularly in early embryonic systems (Xu & Li, 2020; Chavez et al., 2022).

Beyond technical performance, the implications of these findings must be interpreted within a broader ethical and regulatory framework. Although CRISPR-Cas9 demonstrates theoretical potential for preventing monogenic diseases such as Huntington's disease before birth, the persistence of mosaicism, unintended genomic alterations, and unpredictable repair outcomes underscores why germline editing remains unsuitable for clinical application. Ethical concerns—

including the inability of future generations to provide informed consent, the irreversibility of germline modifications, and the risk of exacerbating social inequities—further reinforce the need for caution (De Melo-Martín & Rosenwaks, 2021; Shukal, 2025). Consequently, international scientific bodies continue to support tightly regulated research while maintaining strong prohibitions against reproductive uses of human embryo editing (Vassena et al., 2016; Piergentili et al., 2021).

In this context, the present study contributes to the growing consensus that CRISPR-based germline editing should currently be regarded as an experimental and exploratory research tool rather than a viable clinical intervention. Advances in alternative genome-editing strategies, such as base editing and prime editing, along with improved delivery timing and control of DNA repair pathways, may eventually mitigate some of the risks identified here. Until such technologies demonstrate consistently high precision, minimal unintended effects, and robust safety profiles, germline genome editing should remain confined to non-reproductive research aimed at advancing fundamental understanding of human development and genome repair mechanisms.

Overall, this study supports a cautious and evidence-based perspective on the application of CRISPR-Cas9 in human embryos. While the elimination of inherited diseases at the embryonic stage remains a compelling scientific goal, current technical and ethical limitations necessitate restraint. Continued interdisciplinary collaboration among scientists, clinicians, ethicists, and policymakers will be essential to determine whether—and under what conditions—germline genome editing might one day be responsibly integrated into medical practice.

CONCLUSIONS

This study demonstrates that CRISPR-Cas9 gene editing has the theoretical capacity to correct pathogenic mutations associated with Huntington's disease within a human embryo model. Simulation-based analysis indicates that precise correction of the expanded *HTT* CAG repeat is achievable in a subset of embryos and may partially improve early developmental outcomes and restore normal Huntingtin protein expression patterns. These findings reinforce the conceptual potential of genome editing as a strategy for preventing monogenic inherited diseases before birth. However, the results also reveal substantial technical limitations that currently preclude clinical application. The high prevalence of mosaic editing outcomes, together with the occurrence of unintended off-target and on-target genomic alterations, highlights unresolved safety risks inherent to CRISPR-Cas9–mediated germline editing. Moreover, improvements in early developmental indicators such as blastocyst formation cannot be equated with long-term embryonic health, developmental normality, or clinical safety.

In conclusion, while CRISPR-based germline editing represents a promising scientific advance, it remains an experimental and speculative approach rather than a viable medical intervention. Significant technological refinement, comprehensive genome-wide safety assessment, and robust ethical and regulatory frameworks are essential prerequisites before any consideration of reproductive application. Until such conditions are met, research on human embryo genome editing should continue to prioritize safety, transparency, and ethical responsibility while advancing fundamental understanding of early human development and genome repair mechanisms.

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